One Mission, Inspired by Patients: Defeat Cancer.™

September 17, 2021

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OPENING REMARKS

Steve L. Hoerter

President and Chief Executive Officer

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TODAY'S AGENDA

OPENING REMARKS
Steve L. Hoerter
President and Chief Executive Officer

REBASTINIB DATA UPDATE FROM PHASE 1B/2 STUDY IN COMBINATION WITH PACLITAXEL IN PLATINUM RESISTANT OVARIAN CANCER (PROC)
Robert L. Coleman, M.D., FACOG, FACS
Gynecologic Oncologist and Chief Scientific Officer for US Oncology Research

UNMET MEDICAL NEED AND EXPECTED MILESTONES
Matthew L. Sherman, M.D.
Executive Vice President and Chief Medical Officer

REBASTINIB Q&A

VIMSELTINIB DATA UPDATE FROM THE PHASE 1/2 STUDY IN TENOSYNOVIAL GIANT CELL TUMOR (TGCT)
William D. Tap, M.D.
Chief of the Sarcoma Medical Oncology Service at Memorial Sloan Kettering Cancer Center

VIMSELTINIB PHASE 3 MOTION STUDY
Matthew L. Sherman, M.D.
Executive Vice President and Chief Medical Officer

TGCT MARKET DYNAMICS AND VIMSELTINIB OPPORTUNITIES
Daniel C. Martin
Senior Vice President and Chief Commercial Officer

VIMSELTINIB Q&A

CLOSING REMARKS
Steve L. Hoerter
President and Chief Executive Officer

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REBASTINIB DATA UPDATE FROM PHASE 1B/2 STUDY IN COMBINATION WITH PACLITAXEL IN PROC

Robert L. Coleman, M.D., FACOG, FACS

Gynecologic Oncologist and Chief Scientific Officer for US Oncology Research

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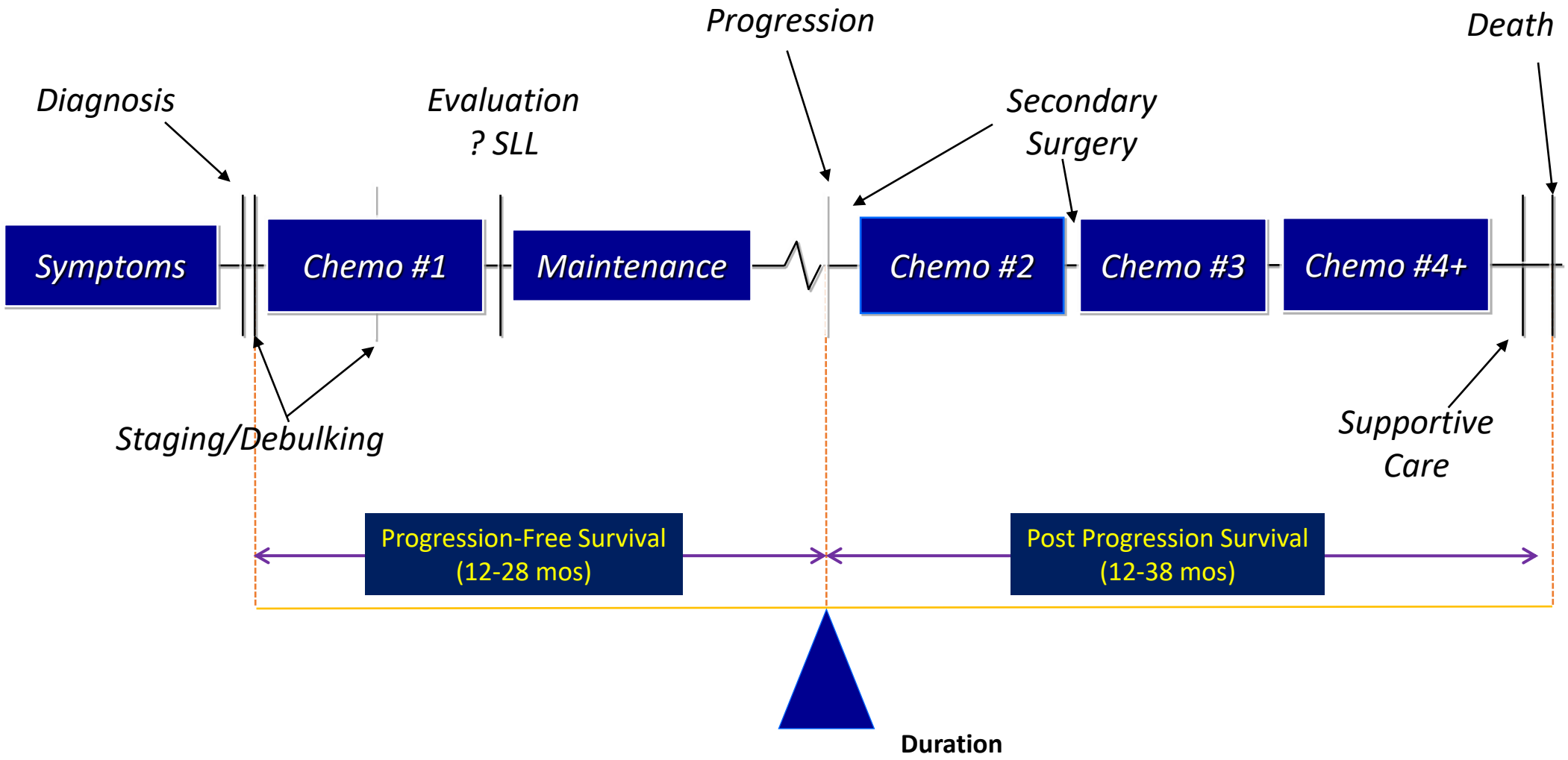
OVARIAN CANCER: NATURAL HISTORY



SLL=second look laparoscopy

MOVING BEYOND THE PLATINUM-SENSITIVE /-RESISTANT PARADIGM

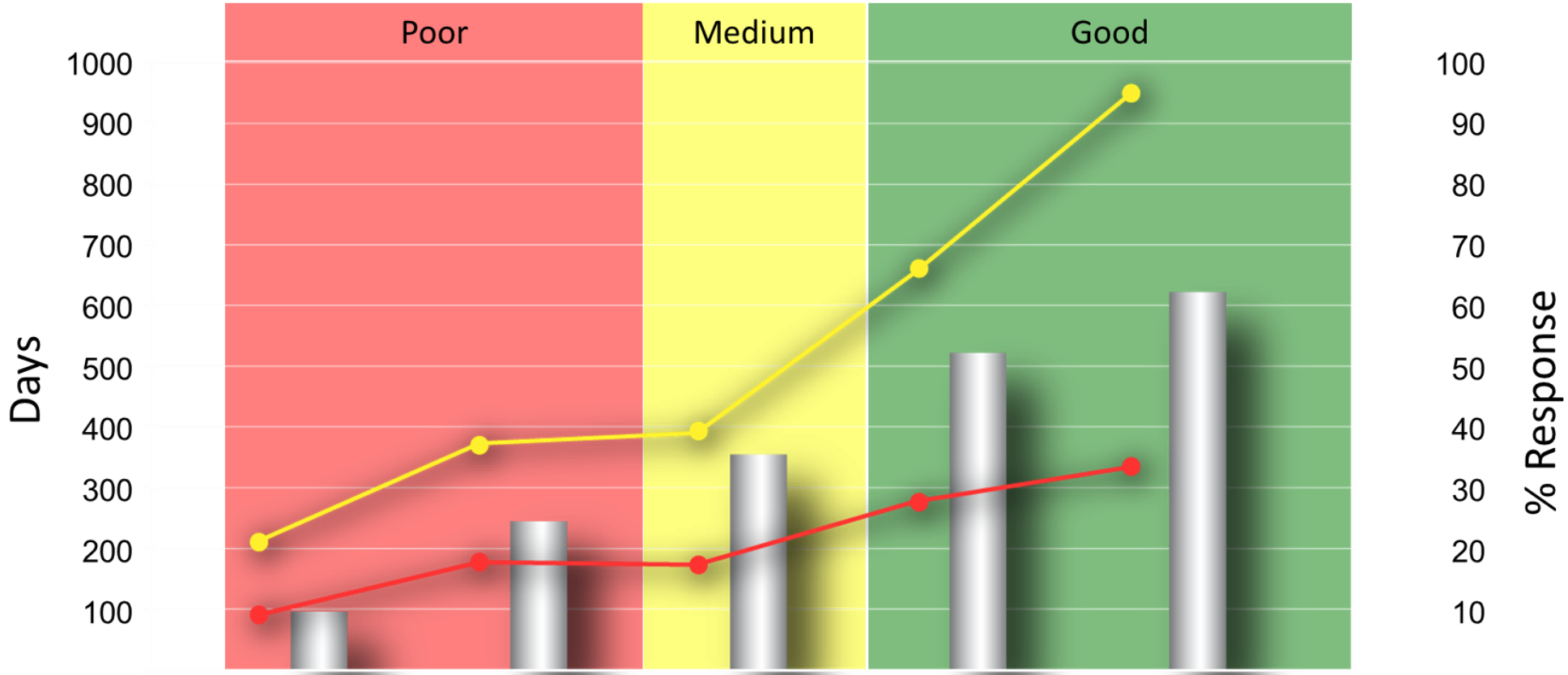
An emerging classification system for recurrent disease based on an increased understanding of the biology of ovarian cancer

Emerging new multiplex classification system

Histology	Molecular Signature	Treatment-Free Interval	Number of Prior Chemotherapy Regimens
1. HGSC/ endometrioid	1. *BRCA*mut	1. <3 months	1. ≤3
2. Other, specify	2. *BRCA*-like	2. 3-12 months	2. >3
	3. Other, specify	3. >12 months	

HGSC=high-grade serous carcinoma. Alvarez RD, et al. *Gynecol Oncol.* 2016;141(3):405–409.

TREATMENT FREE INTERVAL AND ORR, PFS, AND OS



PROGRESSION	0-3 Prog	0-3 Non PD	3-12 mos	12-18 mos	18+ mos
PFS (days)	90	176	174	275	339
OS (days)	217	375	375	657	957
Response (%)	9	24	35	52	62

PD=progressive disease; Prog=progression; PFS=progression-free survival; ORR=overall response rate; OS=overall survival; Lauraine, Proc ASCO #829, 2002

UNMET MEDICAL NEED: TREATMENT EXPECTATIONS

TABLE 2. Efficacy of all lines of therapy in the platinum-resistant/refractory setting (total lines therapy = 689)

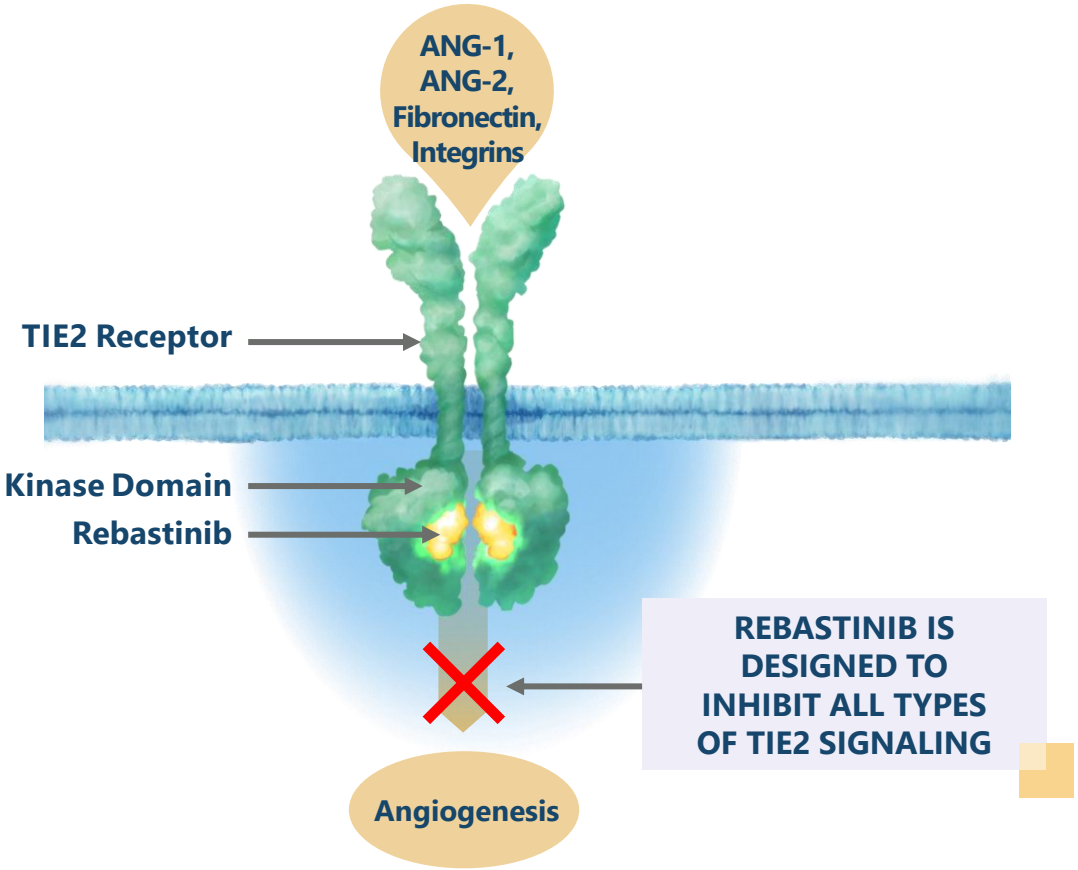
	Line of Therapy After Platinum Resistance									
	First		**Second**		**Third**		**Fourth**		**Fifth+**	
n	274		196		127		62		30	
Radiological response rate (CR + PR), %	15.7	12-21%[1]	8.1	5-13%[1]	3.1	1-8%[1]	1.6	0-8%[1]	0	
Clinical benefit rate (CR, PR + SD), %	36.9		30.6		18.1		17.7		3.3	
Serological response rate, %	49.3		37.1		32.2		23.7		13.3	
PFI, median (95% CI), wk	18 (15–21)		16 (14–18)		13 (10–16)		13 (8–17)		8 (7–9)	
OS, median (95% CI), wk	61 (53–69)		48 (40–56)		40 (33–47)		38 (22–53)		26 (21–31)	

CR=complete response; OS=overall survival; PFI=progression-free interval; PR=partial response; SD=stable disease; wk=weeks; (1) Calculated using modified Wald method.

REBASTINIB

A HIGHLY POTENT AND SELECTIVE TIE2 INHIBITOR

TIE2 SIGNALING ACTS AS A REGULATOR OF TUMOR ANGIOGENESIS, INVASIVENESS, AND METASTASIS



- Rebastinib is a first-in-class investigational, orally administered, potent, and selective switch-control inhibitor of the TIE2 kinase
- TIE2 is the receptor for angiopoietins ANG-1 and ANG-2, an important family of vascular growth factors
- TIE2 receptors are expressed on endothelial cells and angiogenic macrophages, promoting the survival, maturation, and functional integrity of the vasculature; they play an important role in regulating tumor angiogenesis, invasiveness, and metastasis
- Rebastinib binds potently into the switch pocket of TIE2, stabilizing the inhibitory switch and displacing the activation switch to block TIE2 signaling[1]

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Notes: TIE2=TEK tyrosine kinase; (1) Harney AS, et al. *Mol Cancer Ther*. 2017;16:2486.

STUDY DESIGN

PART 1 | **PART 2** (Simon Two-Stage Design)

Part 1 enrolled patients with locally advanced or metastatic solid tumors for which paclitaxel was considered appropriate therapy

Rebastinib
50 mg BID + Paclitaxel[1]
(n = 24)

Rebastinib
100 mg BID + Paclitaxel[1]
(n = 19)

Rebastinib
50 mg BID + Paclitaxel
Phase 2 Dose

Stage 1 *(n ≤ 18 per cohort)*

Stage 2 *(n = up to 33 per cohort)*

- **Cohort 1**[2] | Triple Negative Breast Cancer
- **Cohort 2**[2] | Inflammatory Breast Cancer
- **Cohort 3** | Platinum-resistant Ovarian Cancer
- **Cohort 4** | Endometrial Cancer
- **Cohort 5**[2] | Gynecological Carcinosarcoma

If >5 responses in Stage 1, enroll additional patients in Stage 2 (up to 33 patients per cohort)
If ≤5 responses, discontinue the cohort

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Notes: BID=twice daily; (1) 80 mg/m2 IV infusion over 60 minutes weekly (day 1, day 8, and day 15 of repeated 28-day cycles); (2) Triple negative breast cancer, inflammatory breast cancer, and gynecological carcinosarcoma cohorts did not advance to Stage 2.

PATIENT DEMOGRAPHICS AND DISPOSITION

n = 38
SAFETY POPULATION

Discontinued due to unrelated AE (n = 1)[1]
Withdrew consent (n = 1)[1]
Did not meet eligibility criteria (n = 2)[2]

n = 34
mITT POPULATION

60 YEARS
MEDIAN AGE
of Patients
(range 36-76)

HISTOLOGY

34 (89%)[3]
High-grade serious

2 (5%)
Mixed

1 (3%)
Endometrioid

1 (3%)
Seromucinous

4 Median Number of **PRIOR REGIMENS** (range 2–7)

15 (39%)
2–3 Regimens

23 (61%)
≥4 Regimens

8 (21%) **BRCA+**

PRIOR THERAPY TYPE

38 (100%)
Paclitaxel

33 (87%)
Bevacizumab

26 (68%)
Anti-PARP

8 (21%)
Other

Notes: AE=adverse event; BID=twice daily; BRCA=breast cancer gene; mITT=modified intent-to-treat; PARP=poly adenosine diphosphate-ribose polymerase; (1) Patients who discontinued due to withdrawal of consent or an unrelated AE were excluded because they did not have a post baseline assessment; (2) Of the 2 patients who did not meet eligibility criteria, 1 had non-measurable disease at baseline and the other did not have ovarian cancer; (3) Includes one patient whose histology was classified as "Other, high-grade serous".

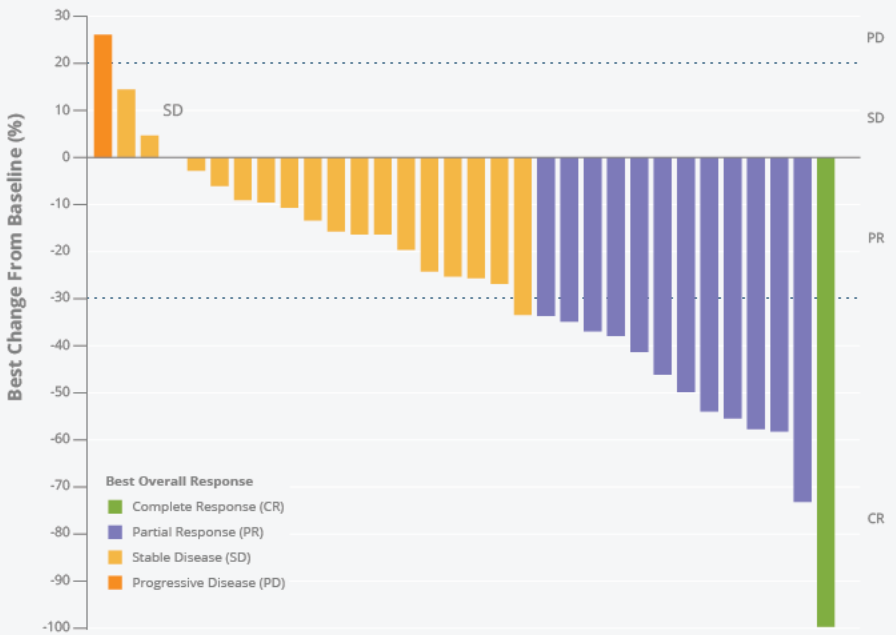
ENCOURAGING ANTI-TUMOR ACTIVITY

38% ORR[1,2] (confirmed and unconfirmed)

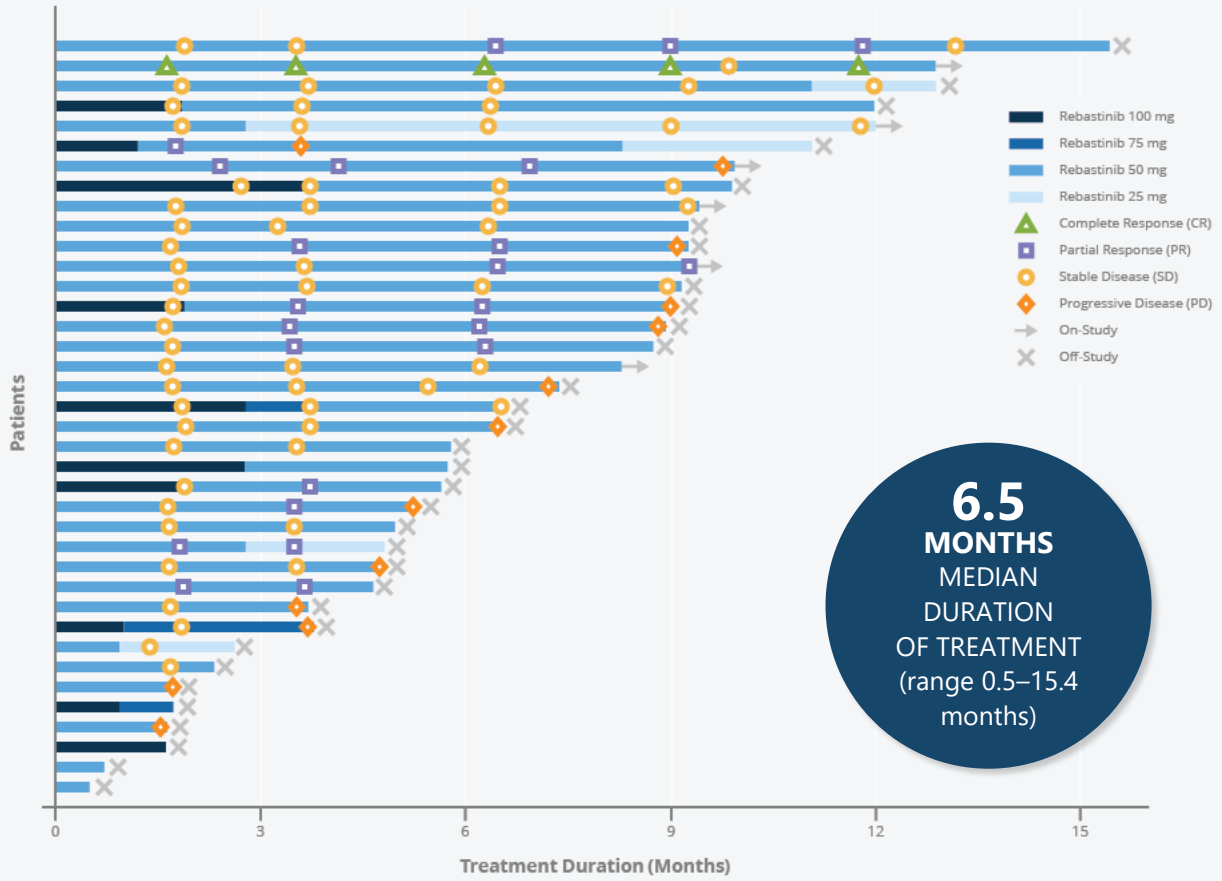
29% ORR[1,2] (confirmed)

76% $CBR_{16\ weeks}$[3]

Best Percent Change from Baseline in Tumor Size[4,5]



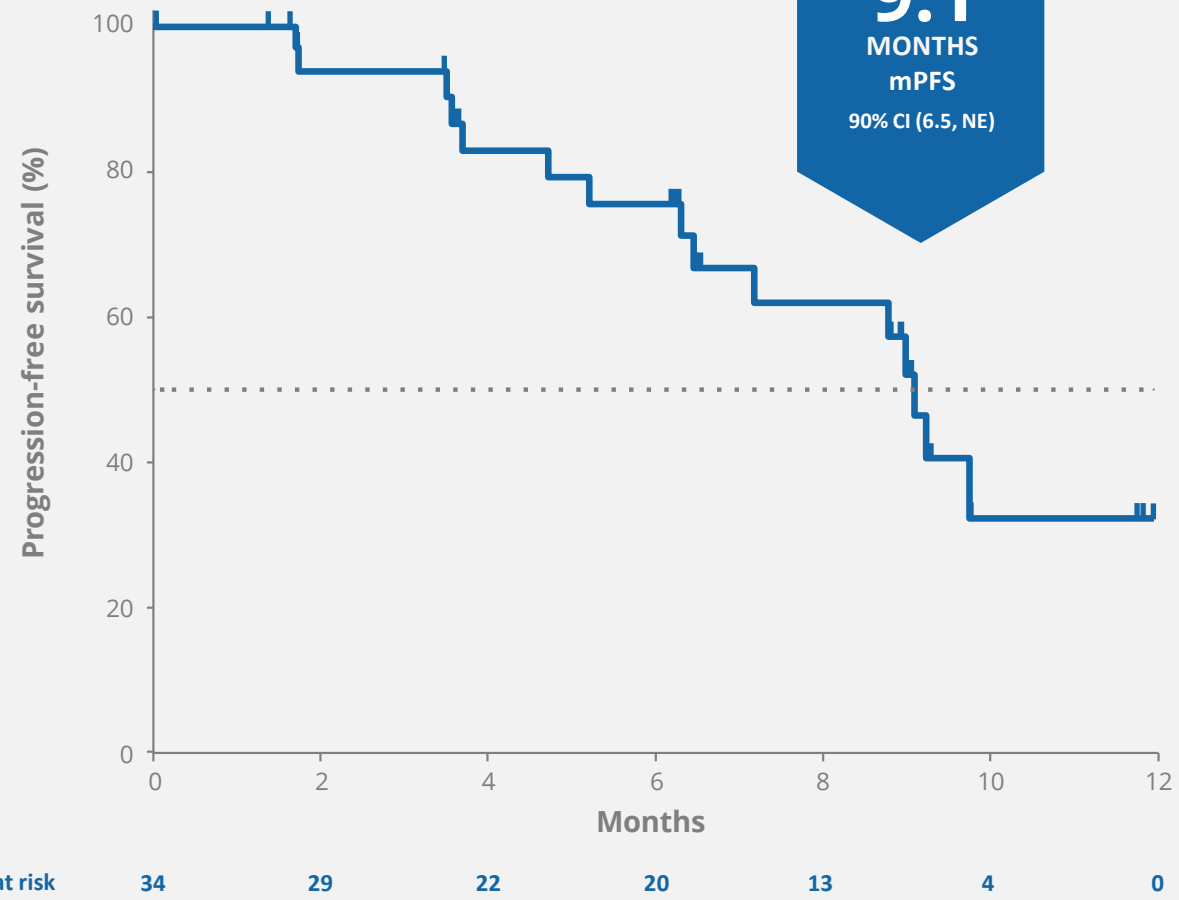
Time on Treatment[2]



6.5 MONTHS MEDIAN DURATION OF TREATMENT (range 0.5–15.4 months)

Notes: Data presented at the ESMO Congress 2021; Results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; CBR=clinical benefit rate; ORR=objective response rate; (1) Includes 13 patients with objective response (10 confirmed, 3 to be confirmed at future follow-up), 18 stable disease, and 1 progressive disease; (2) Overall, 13 patients (34%) discontinued due to radiological PD, 9 patients (24%) discontinued due to an AE, 7 patients (18%) discontinued due to clinical PD, 2 patients (5%) chose to withdraw, and 1 patient (3%) died due to causes unrelated to rebastinib; (3) Clinical benefit rate at 16 weeks was defined as overall response of CR, PR, or SD according to RECIST v1.1 at the 16-week response assessments, respectively; (4) Patients with ≥1 post baseline radiological assessment are shown (n = 32); plot includes confirmed and unconfirmed responses; (5) Dotted lines denote 30% decrease and 20% increase in tumor size cutoffs for PR and PD, respectively.

PROMISING PROGRESSION-FREE SURVIVAL



mPFS of 9.1 months, ORR of 38%[1] (confirmed and unconfirmed), and 29%[1] (confirmed) were promising when considering benchmark data of single agent paclitaxel in PROC setting

BENCHMARK DATA[2–4]

mPFS	**ORR**
3-4 months	15%–25%

Notes: Data presented at the ESMO Congress 2021; Results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; CI= confidence interval; mPFS=median progression-free survival; NE=non-estimable; ORR=overall response rate; PROC=platinum-resistant ovarian cancer; (1) Includes 13 patients with objective response (10 confirmed, 3 to be confirmed at future follow-up), 18 stable disease, and 1 progressive disease; (2) Poveda AM, et al. *J Clin Oncol.* 2015;33:3836–38; (3) Oza A, et al. *Gynecol Oncol.* 2018;149:275–82; (4) Matulonis UA, et al. *Gynecol Oncol.* 2019;152:548–53.

ENCOURAGING TOLERABILITY PROFILE

Table 5. Summary of treatment-emergent AEs ≥15% regardless of relatedness (n = 38)

Preferred term	Any grade	Grade 3	Preferred term	Any grade	Grade 3
Fatigue	22 (58%)	3 (8%)	Hypomagnesemia	8 (21%)	0
Alopecia	16 (42%)	1 (3%)[1]	Urinary tract infection	8 (21%)	1 (3%)
Edema peripheral	15 (39%)	2 (5%)	Abdominal distension	7 (18%)	0
Dry mouth	14 (37%)	0	Anemia	7 (18%)	1 (3%)
Nausea	14 (37%)	1 (3%)	Decreased appetite	7 (18%)	0
Peripheral sensory neuropathy	14 (37%)	0	Hypokalemia	7 (18%)	1 (3%)
Constipation	12 (32%)	0	Vomiting	7 (18%)	1 (3%)
Diarrhea	12 (32%)	2 (5%)	Arthralgia	6 (16%)	0
Hypertension	12 (32%)	3 (8%)	Cough	6 (16%)	0
Abdominal pain	11 (29%)	2 (5%)	Dry eye	6 (16%)	0
Muscular weakness	10 (26%)	3 (8%)[2]	Headache	6 (16%)	0
Stomatitis	10 (26%)	0	Nail discoloration	6 (16%)	0
Dyspnea	9 (24%)	1 (3%)	Pain in extremity	6 (16%)	1 (3%)
Dizziness	8 (21%)	0			

- Most AEs reported were Grade ≤2
- Four patients (11%) experienced five serious AEs at least possibly related to rebastinib:
  - Grade 3 reversible muscular weakness (n = 2; 5%, occurred at 50 mg and 75 mg BID)
  - Grade 2 constipation (n = 1; 3%)
  - Grade 3 fatigue (n = 1; 3%)
  - Grade 3 urinary tract infection (n = 1; 3%)

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Notes: Data presented at the ESMO Congress 2021; Results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; AE=adverse event; BID=twice daily; CTCAE=common terminology criteria for adverse events; SAE=serious AE; (1) Grade 3 alopecia is not in CTCAE, site queried and updated to Grade 2; (2) One patient had Grade 3 muscular weakness that was considered related to rebastinib but was not entered as an SAE. This event occurred at 100 mg BID.

PROMISING RESULTS

The updated safety and efficacy of rebastinib at 50 mg BID in combination with paclitaxel shows encouraging results in heavily pretreated patients with PROC, with an acceptable safety profile, supporting further development.

MEDIAN PFS

9.1
MONTHS

44% of events

CLINICAL BENEFIT RATE

76%

at 16 weeks[2]

OBJECTIVE RESPONSE RATE[1]

38%
(confirmed and unconfirmed)

29%
(confirmed)

CA-125 RESPONSE

73%

occurred in 19/26 patients

MEDIAN DURATION OF TREATMENT

6.5
MONTHS

range 0.5–15.4 months

A MANAGEABLE SAFETY PROFILE

Most AEs reported were Grade ≤2

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UNMET NEED AND EXPECTED MILESTONES

Matthew L. Sherman, M.D.

Executive Vice President and Chief Medical Officer

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PLATINUM-RESISTANT OVARIAN CANCER

LIMITED TREATMENT OPTIONS WITH POOR OUTCOMES

Disease Summary

- 22,000 incident cases a year in women in the U.S.[1]
- In 2020, ~14,000 women died from ovarian cancer in the U.S.[1]

Unmet Medical Need

- Vast majority of patients experience disease recurrence
- Patients that experience disease recurrence eventually develop platinum-resistant ovarian cancer (PROC)
- Outcomes are particularly poor for patients with PROC, driving the need for more effective therapies

Incidence In Ovarian Cancer ~22,000 in the U.S.[1] and comparable incidence in Europe

Platinum-based chemotherapy +/- Bev/PARPi maintenance

1st Line PROC ~6,000 in the U.S.[2] and comparable incidence in Europe

2nd Line PROC

3rd Line PROC

Median survival of PROC is <12 months[3]

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Notes: PARPi=poly-ADP ribose polymerase inhibitor; (1) ACS Cancer Facts & Figures 2020; (2) Internal Deciphera estimates based on market research and secondary data; estimates are inherently uncertain; (3) Tinker D. et al. Gynecol Oncol. 2014;133(2):624-631.

PROMISING RESULTS SUPPORT FURTHER DEVELOPMENT

The updated safety and efficacy of rebastinib at 50 mg BID in combination with paclitaxel shows encouraging results in heavily pretreated patients with PROC, with an acceptable safety profile, supporting further development.

Pivotal Phase 3 study in PROC is anticipated to start in 2022, subject to discussions with health authorities

MEDIAN PFS

9.1
MONTHS

44% of events

CLINICAL BENEFIT RATE

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at 16 weeks[2]

OBJECTIVE RESPONSE RATE[1]

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REBASTINIB Q&A

VIMSELTINIB DATA UPDATE FROM THE PHASE 1/2 STUDY IN TGCT

William D. Tap, M.D.

Chief of the Sarcoma Medical Oncology Service at Memorial Sloan Kettering Cancer Center

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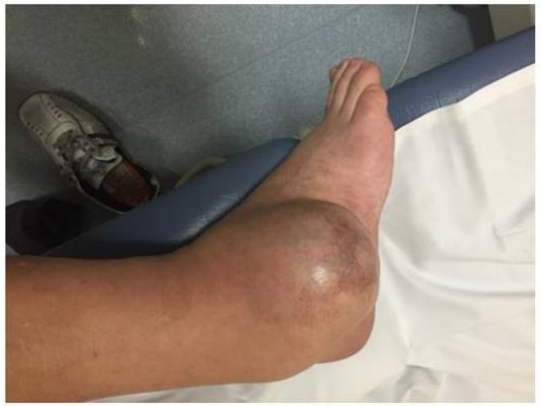
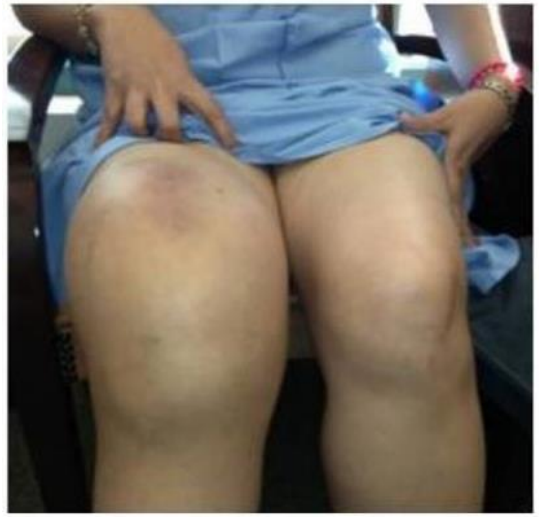
TENOSYNOVIAL GIANT CELL TUMOR (TGCT)

A LOCALLY AGGRESSIVE TUMOR ASSOCIATED WITH SUBSTANTIAL MORBIDITY





Disease Burden and Unmet Need for TGCT Patients

Disease characteristics	• Typically occurs in people 30-50 years old[1] • Genetic translocation causes overproduction of CSF1, triggering migration of inflammatory cells including CSF1R-expressing tumor-associated macrophages (TAMs) to tumor sites[2]
Diagnosis	Due to the slow-growing nature of TGCT and similar symptoms to other conditions like sports injuries and arthritis, patients may have a longer path to diagnosis
Common locations[3]	Knees | Hips | Ankles | Elbows | Shoulders
Patient burden	In the TOPP registry[4], patients at baseline commonly reported multiple symptoms, including pain (78%), limited function (64%), swelling (61%), stiffness (55%), and use of analgesics (15%)[5]

Unmet need	• Surgical resection is standard treatment
	• High rate of recurrence in diffuse TGCT
	• CSF1R inhibition has demonstrated clinical benefit in diffuse TGCT
	• Pexidartinib is only approved agent for TGCT patients no longer amenable to surgery (FDA approval in August 2019)
	  – FDA label includes boxed warning, REMS, and intensive liver monitoring due to off-target hepatotoxicity risks
	  – The EMA adopted the decision of refusal of the Turalio MAA in November 2020
	• Unmet medical need for effective CSF1R inhibitor with favorable safety/tolerability profile for TGCT patients

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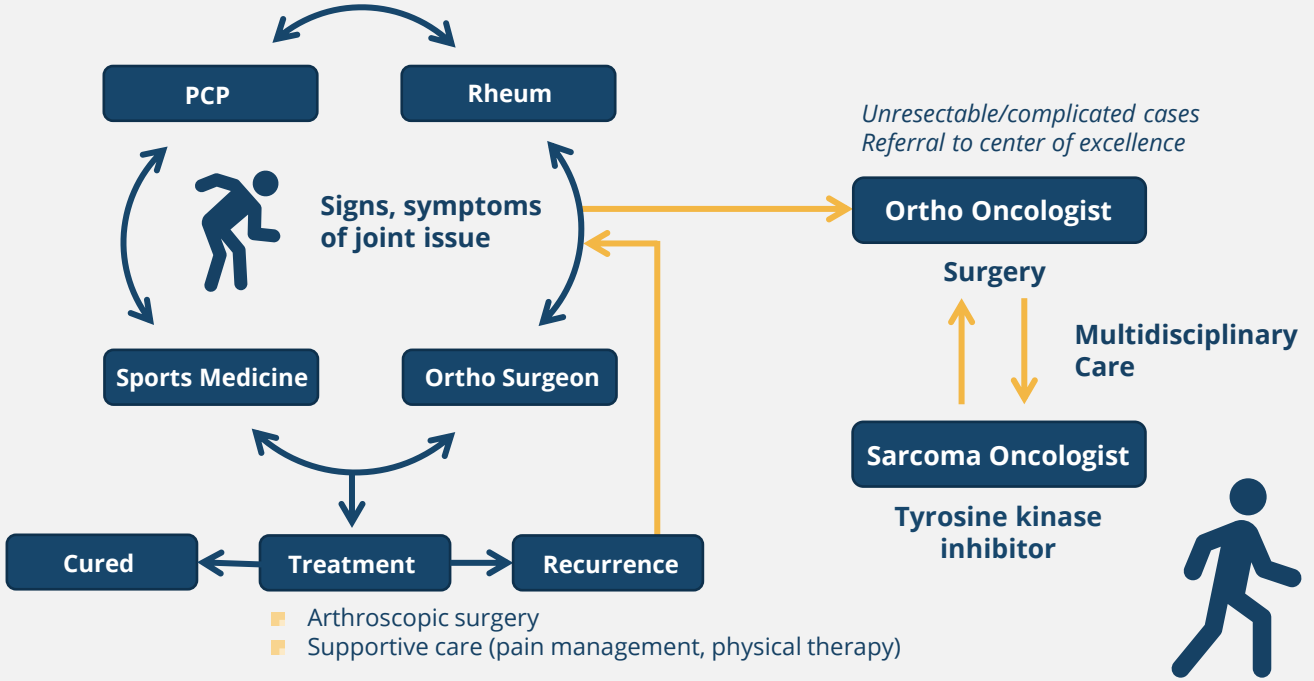
Ankle image courtesy of Tap WD, et al. ASCO 2018, abstract 11502; Knee image courtesy of Tap WD, et al. N Engl J Med. 2015;373:428-437.
Notes: CSF1=colony-stimulating factor 1; CSF1R=colony-stimulating factor 1 receptor; EMA=European Medicines Agency; FDA=U.S. Food and Drug Administration; MAA=Marketing Authorisation Application; REMS=Risk Evaluation and Mitigation Strategy; TGCT=tenosynovial giant cell tumor; (1) Mastboom et al. Acta Orthopaedica. 2017;88:688-694; (2) West et al. Proc Natl Sci USA. 2006; 103:690-695; (3) Common locations are specific to diffuse TGCT; (4) The TOPP registry was a multinational, multicenter, prospective observational study involving 12 tertiary sarcoma centers in 7 European countries, and 2 US sites; (5) Patients experienced more than or equal to 3 symptoms (52%).

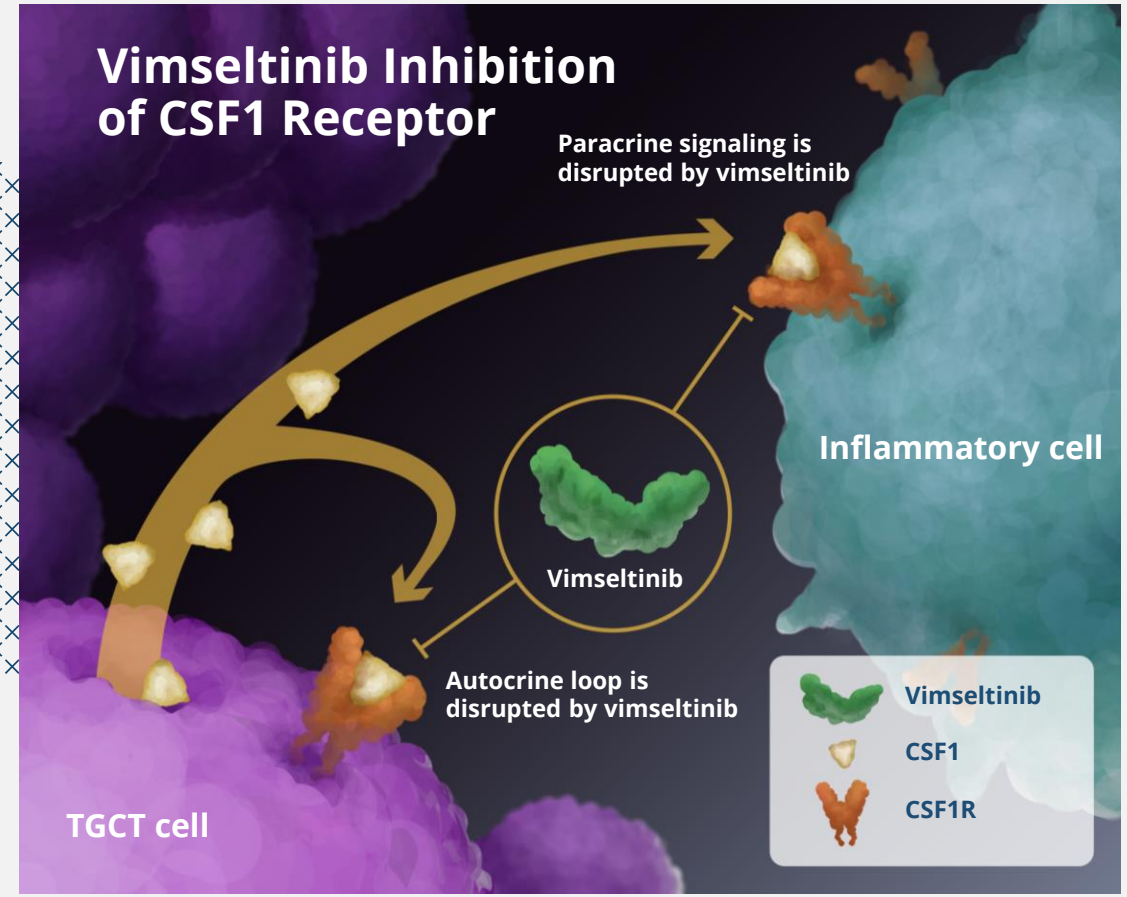
PATIENT JOURNEY OF TGCT PATIENT NOT AMENABLE TO SURGERY

TGCT PATIENT JOURNEY



Source: Spierenburg, G. The Diffuse-Type TGCT Patient Journey: A Prospective Multicenter Study. Poster presented at: 2020 Connective Tissue Oncology Society (CTOS) Virtual Annual Meeting; November 18 – 21, 2020.
Notes: Ortho=orthopedic; PCP=primary care physician; Rheum=rheumatologist; TGCT=tenosynovial giant cell tumor.

POTENTIAL BEST-IN-CLASS CSF1R INHIBITOR IN DEVELOPMENT



- Vimseltinib is an oral, switch-control TKI specifically designed to selectively and potently inhibit CSF1R
- Phase 1/2 study is ongoing in patients with solid tumors and TGCT
  - Enrollment complete for Cohort A (patients with no prior anti-CSF1/CSF1R therapy)
  - Enrollment ongoing for Cohort B (patients with prior anti-CSF1/CSF1R therapy)
- The recommended Phase 2 dose for vimseltinib in TGCT patients was determined to be 30 mg twice weekly

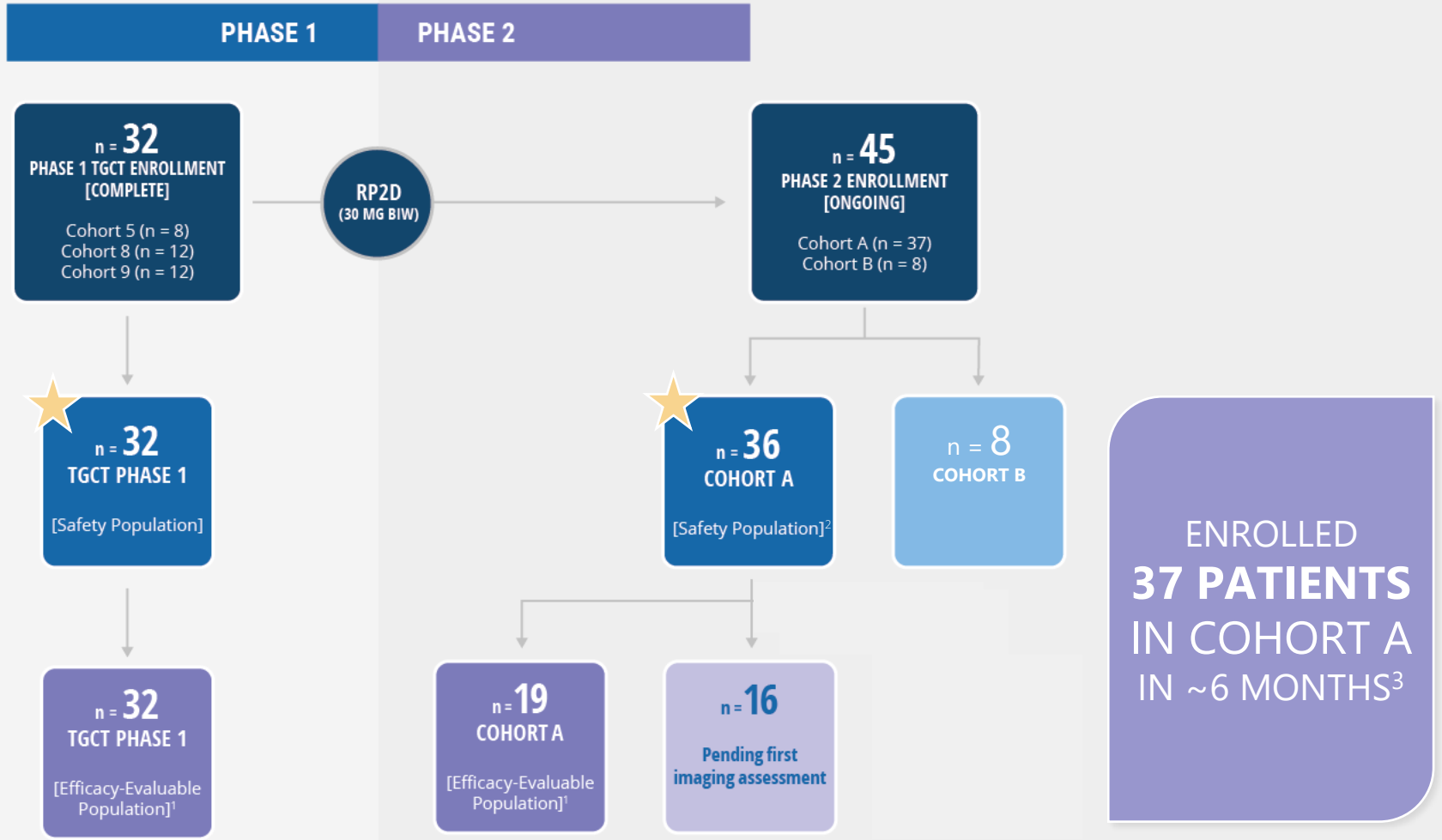
Notes: CSF1R=colony-stimulating factor 1 receptor; TGCT=tenosynovial giant cell tumor; TKI=tyrosine kinase inhibitor.

STUDY DESIGN

Enrollment in Phase 1 dose escalation is complete and ongoing in Phase 2 at the RP2D (30 mg twice weekly with no loading dose).

	Loading doses	Dose
Cohort 5	30 mg QD x 5 days	30 mg twice weekly
Cohort 8	30 mg QD x 3 days	10 mg QD
Cohort 9	20 mg QD x 3 days	6 mg QD
Expansion	NA	30 mg twice weekly

★ Data presented at the ESMO Congress 2021 is from the Phase 1 dose escalation portion of the study and from Cohort A in the Phase 2 expansion portion of the study.



Notes: Data presented at the ESMO Congress 2021; Results are reported for patients with TGCT with a data cutoff of June 7, 2021; EDC=electronic data capture; QD=once daily; RP2D=recommended Phase 2 dose; TGCT=tenosynovial giant cell tumor; (1) At least one post-baseline efficacy assessment; (2) 1 patient withdrew prior to evaluation; (3) 1 patient pending dosing data in the database.

BASELINE CHARACTERISTICS

	Phase 1 TGCT patients (n = 32)	Phase 2 Cohort A patients (n = 36)
Median Age, years (range)	51 (23–73)	44 (21–71)
Sex		
Female	17 (53%)	26 (72%)
Male	15 (47%)	10 (28%)
Race		
White	31 (97%)	28 (78%)
Asian	1 (3%)	2 (6%)
Not Reported or Missing	0	6 (17%)
Disease location		
Knee	20 (63%)	20 (56%)
Ankle	5 (16%)	5 (14%)
Hip	4 (13%)	2 (6%)
Foot	1 (3%)	6 (17%)
Other[1]	2 (6%)	3 (8%)
Patients with at least one prior surgery	12 (38%)	32 (89%)
Patients with at least one prior systemic therapy	5 (16%)	2 (6%)
Imatinib or nilotinib	4 (13%)	2 (6%)
Lacnotuzumab (MCS-110)	1 (3%)	0

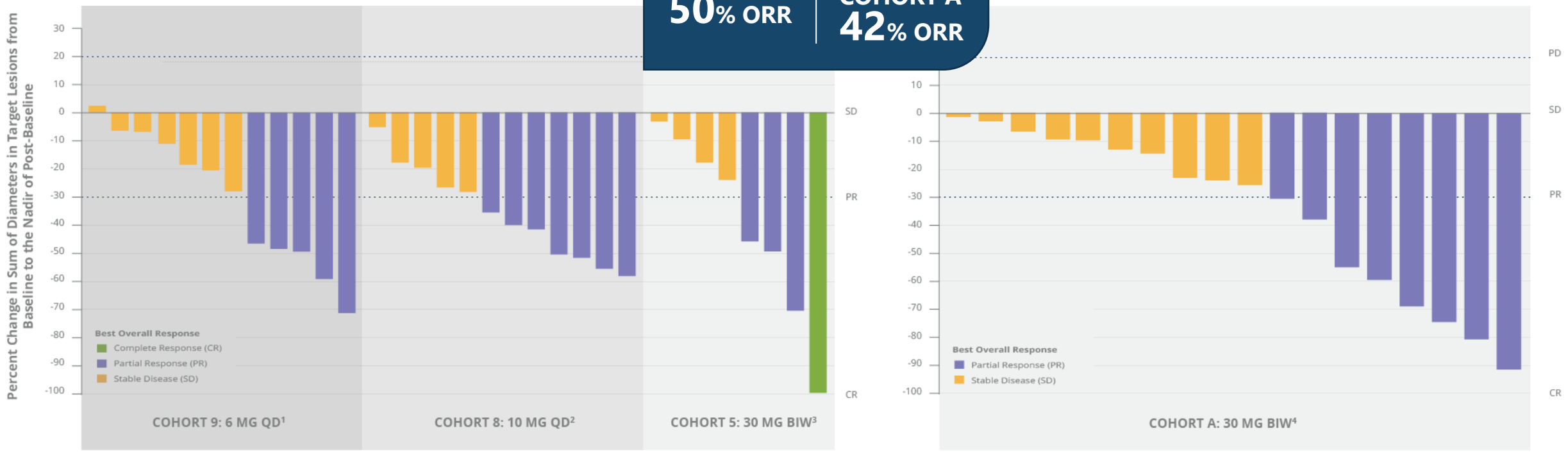
Notes: Data presented at the ESMO Congress 2021; Results are reported for patients with TGCT with a data cutoff of June 7, 2021; TGCT=tenosynovial giant cell tumor; Data are presented as n (%) unless otherwise noted; Percentages might not add up to 100% due to rounding; (1) Other locations include wrist, shoulder, and jaw.

ENCOURAGING ANTI-TUMOR ACTIVITY

47% ORR

Across all dose cohorts of Phase 1 and Phase 2 Cohort A

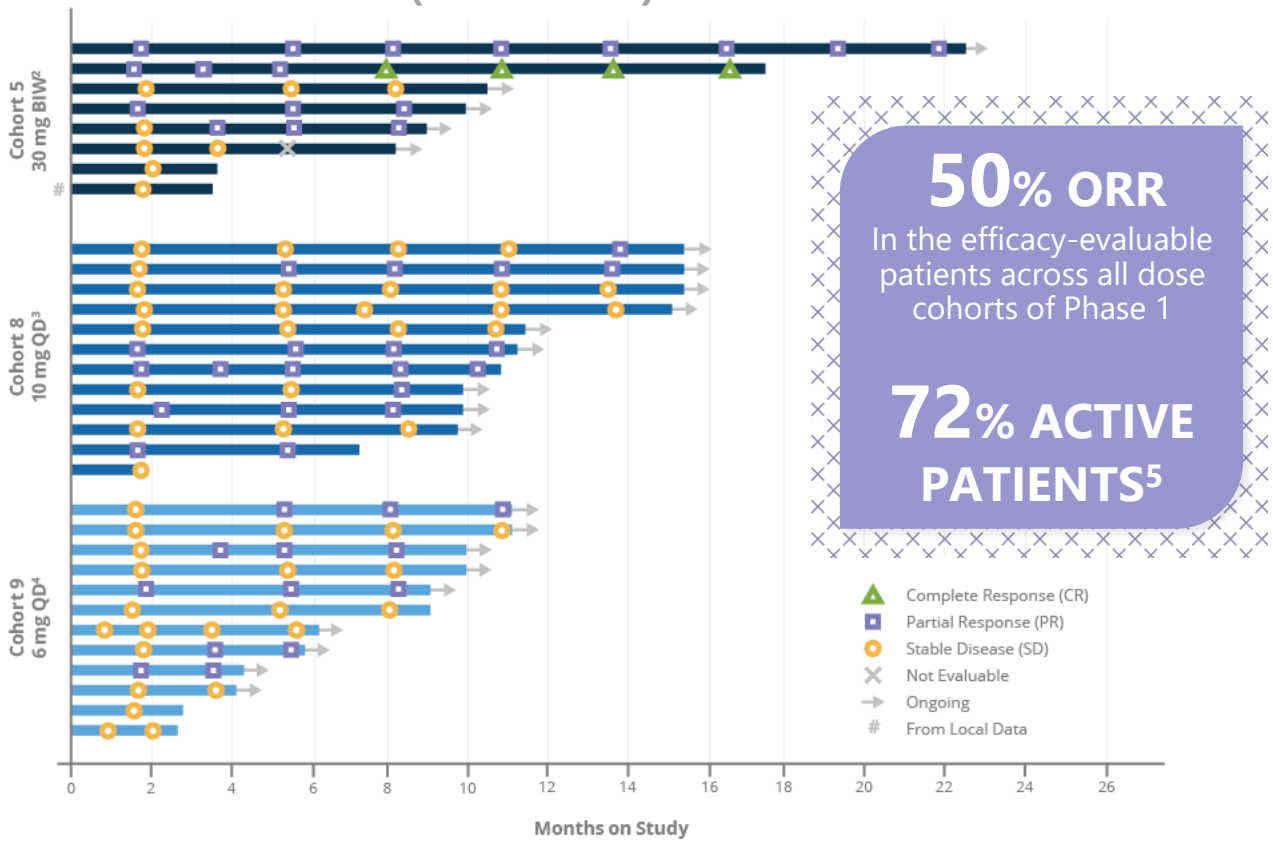
PHASE 1	PHASE 2 COHORT A
50% ORR	42% ORR



Notes: Data presented at the ESMO Congress 2021; results are reported for patients with TGCT with a data cutoff of June 7, 2021; BIW=twice weekly; CR=complete response; ORR=objective response rate; PR=partial response; QD=once daily; SD=stable disease; TGCT=tenosynovial giant cell tumor; (1) After 3-day 20 mg QD loading dose; (2) After 3-day 30 mg QD loading dose; (3) After 5-day 30 mg QD loading dose; (4) No loading dose.

DURABLE RESPONSES TO TREATMENT OBSERVED

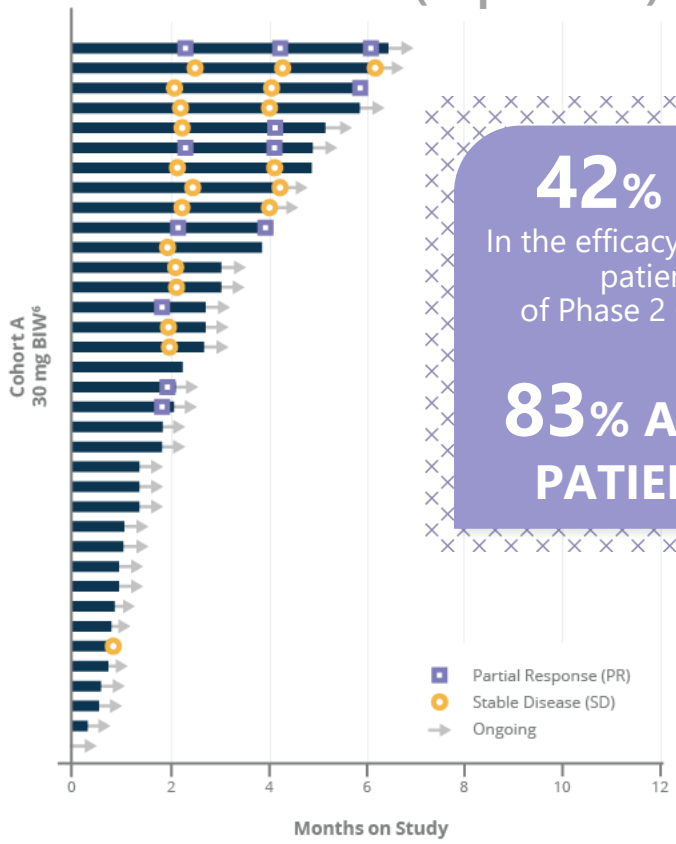
Phase 1 (Escalation)[1]



50% ORR
In the efficacy-evaluable patients across all dose cohorts of Phase 1

72% ACTIVE PATIENTS[5]

Phase 2 – Cohort A (Expansion)[7]



42% ORR
In the efficacy-evaluable patients of Phase 2 Cohort A

83% ACTIVE PATIENTS[5]

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Notes: Data presented at the ESMO Congress 2021; results are reported for patients with TGCT with a data cutoff of June 7, 2021; BIW=twice weekly; ORR=objective response rate; QD=once daily; TGCT=tenosynovial giant cell tumor; #=1 patient had a local assessment for efficacy, but no central assessment was performed; (1) Median duration of treatment of 10.1 months across all phase 1 dose cohorts; (2) After 5-day 30 mg QD loading dose; (3) After 3-day 30 mg QD loading dose; (4) After 3-day 20 mg QD loading dose; (5) Active patients as of data cutoff of June 7, 2021; (5) Active patients as of data cutoff of June 7, 2021; (6) No loading dose; (7) Median duration of treatment of 1.9 months in phase 2 cohort A.

WELL-TOLERATED IN TGCT PATIENTS

TEAEs in ≥15% of Patients Receiving Vimseltinib

Preferred term	Phase 1 Cohort 5 (n = 8)		Phase 1 All Patients[1] (n = 32)		Phase 2 Cohort A[1] (n = 36)	
	All grades	Grade 3/4	All grades	Grade 3/4	All grades	Grade 3/4
Blood CPK increased	7 (88%)	4 (50%)	20 (63%)	10 (31%)	19 (53%)	9 (25%)
Periorbital edema	3 (38%)	0	17 (53%)	0	8 (22%)	0
Fatigue	3 (38%)	0	15 (47%)	0	6 (17%)	0
AST increased	5 (63%)	1 (13%)	14 (44%)	4 (13%)	12 (33%)	0
ALT increased	2 (25%)	0	10 (31%)	1 (3%)	4 (11%)	0
Myalgia	0	0	9 (28%)	1 (3%)	5 (14%)	0
Arthralgia	2 (25%)	0	8 (25%)	1 (3%)	2 (6%)	0
Face edema	0	0	8 (25%)	0	0	0
Headache	3 (38%)	0	8 (25%)	0	10 (28%)	0
Lipase increased	1 (13%)	0	8 (25%)	3 (9%)	4 (11%)	0
Peripheral edema	1 (13%)	0	8 (25%)	0	5 (14%)	0
Pruritus	1 (13%)	0	8 (25%)	0	3 (8%)	0
Amylase increased	1 (13%)	1 (13%)	7 (22%)	2 (6%)	5 (14%)	0
Diarrhea	1 (13%)	1 (13%)	6 (19%)	1 (3%)	2 (6%)	0
Generalized edema	2 (25%)	0	6 (19%)	0	0	0
Hypertension	0	0	6 (19%)	2 (6%)	1 (3%)	0
Nausea	2 (25%)	0	6 (19%)	0	8 (22%)	0
Constipation	1 (13%)	0	5 (16%)	0	2 (6%)	0
Parasthesia	0	0	5 (16%)	0	1 (3%)	0
Rash macular	0	0	5 (16%)	0	0	0
Rash maculopapular	0	0	5 (16%)	0	5 (14%)	0
Asthenia	1 (13%)	0	3 (9%)	0	6 (17%)	0

- Majority of the common (≥15%) TEAEs were ≤Grade 2
- Observed transaminase, pancreatic, and CPK enzyme elevations were mostly low grade and not associated with symptoms
- No abnormalities in bilirubin levels reported

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Notes: Data presented at the ESMO Congress 2021; Results are reported for patients with TGCT with a data cutoff of June 7, 2021; Data are presented as n (%) unless otherwise noted; ALT=alanine aminotransferase; AST=aspartate aminotransferase; CPK=creatine phosphokinase; TEAE=treatment-emergent adverse event; (1) TEAE cutoff of ≥15% based on all grades for total Phase 1 and Phase 2 Cohort A.

ENCOURAGING RESULTS SUPPORT FURTHER EVALUATION

- In patients with TGCT not amenable to surgical resection, vimseltinib demonstrated encouraging preliminary efficacy
- Vimseltinib was generally well-tolerated, and the safety profile remains manageable with longer-term follow-up across all Phase 1 dose cohorts and at the recommended Phase 2 dose in Cohort A
- These results support further evaluation of vimseltinib in the MOTION study, a randomized, placebo-controlled, Phase 3 trial in patients with TGCT not amenable to surgical resection

OBJECTIVE RESPONSE RATE

47%

Across all dose cohorts of Phase 1 and Phase 2 Cohort A

DEEPENING AND DURABLE RESPONSES OBSERVED ACROSS ALL DOSE COHORTS OF PHASE 1

ACTIVE PATIENTS

PHASE 1	PHASE 2 COHORT A
72%	83%

NO ABNORMALITIES IN BILIRUBIN LEVELS REPORTED

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Notes: Data presented at the ESMO Congress 2021; results are reported for patients with TGCT with a data cutoff of June 7, 2021; TGCT= tenosynovial giant cell tumor.

VIMSELTINIB PHASE 3 MOTION STUDY

Matthew L. Sherman, M.D.

Executive Vice President and Chief Medical Officer

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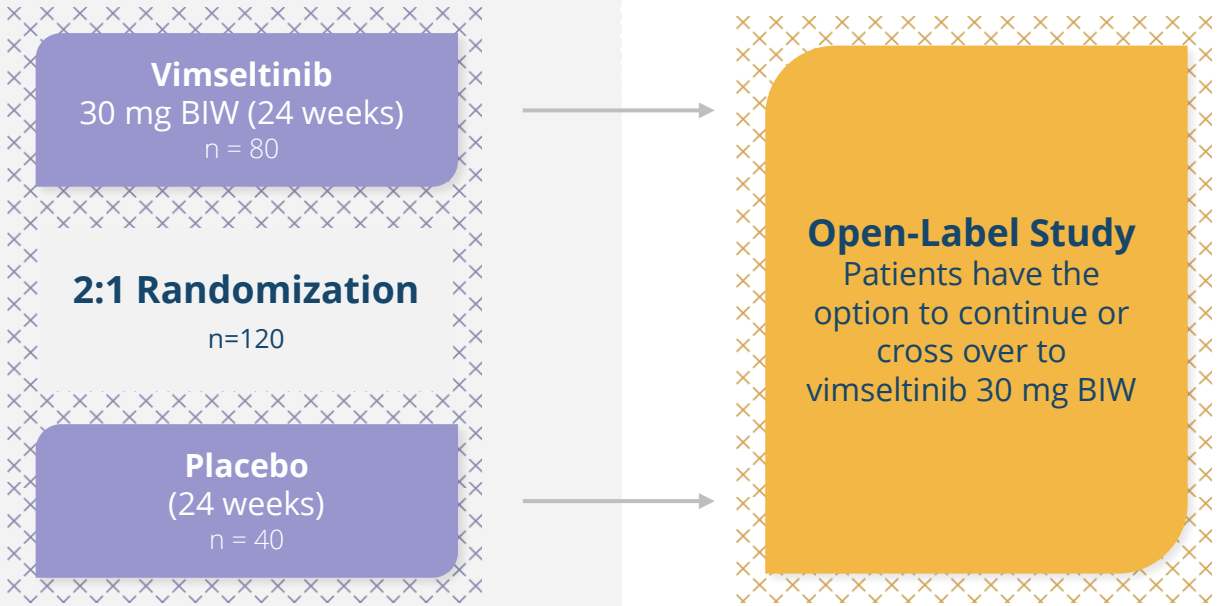
A MULTICENTER, RANDOMIZED, PLACEBO-CONTROLED, DOUBLE-BLIND STUDY

International Study with ~40 Sites

DOUBLE-BLIND PERIOD	OPEN-LABEL PERIOD
Part 1: eligible study participants will be assigned to receive either vimseltinib or matching placebo for 24 weeks.	**Part 2**: a long-term treatment phase in which all participants receive open-label vimseltinib.



Phase 3 Motion Study will assess the efficacy and safety of vimseltinib for the treatment of patients with TGCT not amenable to surgical resection

Primary Endpoint:

- Objective response rate (ORR) at 25 weeks

Key Secondary Endpoints:

- Range of motion (ROM)
- Patient-reported outcomes
- ORR per tumor volume score

STUDY INITIATION IS PLANNED FOR 4Q 2021

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Notes: BIW=twice weekly; TGCT=tenosynovial giant cell tumor.

TGCT MARKET DYNAMICS AND VIMSELTINIB OPPORTUNITIES

Daniel C. Martin

Senior Vice President and Chief Commercial Officer

deciphera™

TENOSYNOVIAL GIANT CELL TUMOR (TGCT)

A SIGNIFICANT OPPORTUNITY EXISTS TO IMPROVE THE LIVES OF TGCT PATIENTS

PCPs, OTHERS → **SURGEONS** → **MEDICAL ONCOLOGISTS**

Diagnosis

~14,000–18,000 patients diagnosed annually in U.S. (localized and diffuse)[1,2]

Literature suggests comparable incidence and recurrence rates in Europe[1,2]

Amenable to surgery (~98%)[2]

Recurrence

~2,000–2,400 patients recur after 1st surgery [1,3]

≥1 additional surgeries

Not amenable to surgery (~2%)[2]

Rx-Treated (not amenable to surgery)

~1,300–1,400 incident Rx-treated patients in the U.S.[1-3]

~8,000 estimated prevalent Rx-eligible patients[1-3]

Notes: PCP=primary care physician; TGCT=tenosynovial giant cell tumor; (1) Mastboom et al. Acta Orthopaedica. 2017;88(6):688-694; (2) Internal Deciphera estimates based on market research and secondary data; estimates are inherently uncertain; (3) Ehrenstein. J Rheumatol. 2017;44(10):1476-1483.

POTENTIAL BEST-IN-CLASS PROFILE

Products Used In TGCT[1]

imatinib

pexidartinib

nilotinib sunitinib

Existing Product Profiles

Imatinib

- Not FDA or EMA approved for TGCT
- Weak CSF1R inhibitor
- Guideline listed based on retrospective data showing 19% ORR[2,3]

Pexidartinib

- FDA approved for TGCT, not approved in EU
- Inhibitor of CSF1R, KIT, FLT3, and PDGFRA/B
- Boxed warning for hepatoxicity, REMS, intensive liver monitoring

Vimseltinib Opportunity

High unmet need

- Lifelong condition with significant morbidity
- HCPs and patients cite desire for highly effective therapy without having to sacrifice safety and tolerability[1]
- No approved therapies ex-US

Potential Best-In-Class Profile[4]

- Highly potent & selective CSF1R inhibitor
- Deep and durable responses
- Limited off-target toxicities with no observed cholestatic hepatoxicity

Strong strategic fit

- TGCT and GIST are sarcomas with overlapping KOLs and call-points
- Significant operational synergies

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Notes: CSF1R=colony-stimulating factor 1 receptor; EMA=European Medicines Agency; FDA=Food and Drug Administration; FLT3=FMS-like tyrosine kinase 3; GIST=Gastrointestinal stromal tumor; HCP=healthcare provider; KIT=KIT prot-oncogene receptor tyrosine kinase; KOL=key opinion leader; PDGFRA/B=platelet derived growth factor A/B; REMS=risk evaluation and mitigation strategy; TGCT=tenosynovial giant cell tumor; (1) Internal Deciphera market research; (2) NCCN Guidelines Version 2.2021 Soft Tissue Sarcoma; (3) Cassier et al Cancer 2012:119:1649-1655; (4) Based on data from phase 1/2 study presented at ESMO Congress 2021 (cut-off date June 7, 2021).

VIMSELTINIB Q&A

CLOSING REMARKS

Steve L. Hoerter

President and Chief Executive Officer

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THANK YOU